## (19) World Intellectual Property Organization International Bureau





(43) International Publication Date 12 May 2005 (12.05.2005)

PCT

(10) International Publication Number WO 2005/042705 A2

(51) International Patent Classification7:

**C12N** 

(74) Agent: DELUCA, Mark; Cozen O'Connor, 1900 Market Street, Philadelphia, PA 19103 (US).

(21) International Application Number:

PCT/US2004/035124

(22) International Filing Date: 22 October 2004 (22.10.2004)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

60/513,489

22 October 2003 (22.10.2003)

(71) Applicant (for all designated States except US): THE TRUSTEES OF THE UNIVERSITY OF PENN-SYLVANIA [US/US]; 3160 Chestnut Street, Suite 200, Philadelphia, PA 19104-6283 (US).

(72) Inventors; and

(75) Inventors/Applicants (for US only): MOURELATOS, Zissimos [GR/US]; 3160 Chestnut Street, Suite 200, Philadelphia, PA 19104-6283 (US). SHARMA, Anup [US/US]; 3160 Chestnut Street, Suite 200, Philadelphia, PA 19104-6283 (US). KIRIAKIDOU, Marianthi [GR/US]; 3160 Chestnut Street, Suite 200, Philadelphia, PA 19104-6283 (US).

(81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD,

MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.

(84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PL, PT, RO, SE, SI,

SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

**Published:** 

without international search report and to be republished upon receipt of that report

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: SHORT INTERFERING RNA AND MICRO-RNA COMPOUNDS AND METHODS OF DESIGNING, MAKING AND USING THE SAME

(57) Abstract: Methods of identifying, designing and synthesizing uniquely targeting siRNA nucleotide sequences for a target mRNA sequence of a target species are disclosed. Methods of identifying, designing and synthesizing miRNA nucleotide sequences that does not function as a siRNA nucleotide sequence for mRNA of a target species are disclosed. Method of inhibiting expression of a target mRNA molecule are disclosed sequence. siRNA molecules including uniquely targeting siRNA molecules and RISCs comprising the same are disclosed. miRNA molecules that do not function as siRNA nucleotide sequences for mRNA of a target species are disclosed.